CIRM Funded Clinical Trials

Phase 1/2 study for autologous human CD34+ hematopoietic stem cells ex vivo transduced with pCCL-CTNS lentiviral vector for treatment of Cystinosis.

Disease Area:	Kidney Failure
Investigator:	Stephanie Cherqui
Institution:	University of California, San Diego
CIRM Grant:	CLIN2-11478
Award Value:	$11,999,944
Trial Sponsor:	University of California, San Diego
Trial Stage:	Phase 1/2
Trial Status:	Not yet recruiting
Targeted Enrollment:	6
ClinicalTrials.gov ID:	NCT03897361

Stephanie Cherqui

Details:
Cystinosis is a rare disease that primarily affects children and young adults, and leads to premature death, usually in early adulthood. Patients inherit defective copies of a gene called CTNS, which results in abnormal accumulation of an amino acid called cystine in all cells of the body. This buildup of cystine can lead to multi-organ failure, with some of earliest and most pronounced effects on the kidneys, eyes, thyroid, muscle, and pancreas. Many patients suffer end-stage kidney failure and severe vision defects in childhood, and as they get older, they are at increased risk for heart disease, diabetes, bone defects, and neuromuscular defects.

This clinical trial will use a gene therapy approach to modify a patient's own blood stem cells with a functional version of the defective CTNS gene. Based on pre-clinical data, the approach is to reintroduce the corrected stem cells into the patient to give rise to blood cells that will reduce cystine buildup in affected tissues.

Design:
This is a Phase I/II clinical trial.

Goal:
Because this is the first time this approach has been tested in patients, the primary goal of the clinical trial is to see if the treatment is safe. In addition, patients will be monitored for improvements in the symptoms of their disease.